Novel Platforms to Enhance In Vivo Delivery of Skeletal Muscle Progenitor Cells from Human Pluripotent Stem Cells

Grant Award Details

Novel Platforms to Enhance In Vivo Delivery of Skeletal Muscle Progenitor Cells from Human Pluripotent Stem Cells

Grant Type: Inception - Discovery Stage Research Projects

Grant Number: DISC1-08823

Project Objective: To explore novel approaches to increase the efficiency of delivering and monitoring SMPCs derived from human induced pluripotent stem cells (hiPSCs)

Investigator:

Name:	April Pyle
Institution:	University of California, Los Angeles
Type:	PI

Disease Focus: Muscular Dystrophy, Skeletal/Smooth Muscle disorders

Human Stem Cell Use: iPS Cell

Award Value: $230,400

Status: Closed

Progress Reports

Reporting Period: Year 2

View Report

Grant Application Details

Application Title: Novel Platforms to Enhance In Vivo Delivery of Skeletal Muscle Progenitor Cells from Human Pluripotent Stem Cells

Public Abstract:

Research Objective

Delivery of muscle stem cells presents a major roadblock for therapy. We explore novel approaches to increase the efficiency of delivering and monitoring muscle stem cells derived from hPSCs.

Impact

Development of enhanced monitoring and delivery platforms will greatly accelerate translational strategies aimed at delivering muscle stem cells for transplantation to patients with muscle disease.

Major Proposed Activities

- Aim 1. Develop a mesoporous silica nanoparticle (MSNP) platform to deliver controlled and localized release of small molecules to enhance SMPCs engraftment in vivo.
- Aim 2. To develop a high throughput system using a multiplexed bioluminescence imaging (BLI) platform to enable evaluation of SMPC survival and engraftment in vivo.

Statement of Benefit to California:

Skeletal muscle wasting disorders including muscular dystrophies, atrophy or aging will affect subsets if not all California (CA) residents during his/her lifetime. Replacement of exhausted muscle stem cells with new stem cells could provide a renewable source of muscle stem cells to extend life span and/or enhance quality of life of citizens of CA. Improved health of CA citizens will also reduce the health care costs associated with muscle disease that occur over the lifetime of CA residents.
